

*In Vivo* Efficacy Fabry Mice:  
“Combination Therapy”

Pre-treat with single  
infusion of  $\alpha$ -galactosidase A

GB3  $\downarrow$

2 WEEKS

NB-DNJ, AMP-DNJ  
or D-t-et-P4  
(IP dose, 1x daily)

→ ↓

4 WEEKS

GB3  $\uparrow$

Study Goal: Evaluate the effect of Substrate Inhibition  
in Reducing GB3 Re-accumulation.

# Fabry Mouse Liver Re-accumulation of GB3 (IP delivery)

FIG. 1B

